Haemophilia outcome measures: Industry’s view

EHC Round Table of Stakeholders
‘Outcome measures in haemophilia’
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ABOUT THE SPEAKER

• Jason Booth, MPH
• Associate Director, Global HEOR Haematology
• Shire
• US
## DISCLOSURES

<table>
<thead>
<tr>
<th>Conflict</th>
<th>Disclosure - if conflict of interest exists</th>
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<tr>
<td>Employee</td>
<td>Shire</td>
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We need to better understand and use health outcome information in haemophilia

• Expectations of people with haemophilia are increasing all over the world and participation in social, work and physical activities is improving\(^1\)

• Annualised Bleed Rate (ABR) is significantly reduced with prophylaxis compared to on demand treatment\(^2\) however ABR doesn’t reflect all disease impacts

• As a result ABR has limited utility in measuring further improved outcomes and current Health-Related Quality of Life measures are of limited use outside a research setting\(^1\)

• With new therapies emerging it is important to develop an individualized view of the impact of therapy and, with support from payers, to use this information to improve standards of care

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New measures should be practical, sensitive and complement existing measures.

- Complementary to standard outcomes, such as ABR\(^1\)
- Aligned with measures meaningful to payers\(^3\)
- Sensitive to small changes that are meaningful to patients\(^1\)
- Encourage and support best practice in patient-centric care\(^1\)
- Practically useful in clinical care AND as a research tool\(^1\)

3. Berger et al. Securing reimbursement for patient centered haemophilia care: major collaborative efforts are needed. *Haematologica* (2016); 101 (3)
Goal Attainment Scaling (GAS) is an established approach to quantifying individual treatment goals\(^1\)

- Shared decision making in the process of setting goals and appropriate therapy is essential for effective patient-centric care\(^5\)
- GAS - A *standardized* approach to *individualized* (patient-centered) goal monitoring, that is often done informally in clinical care\(^1\)
- Engages the patient/caregiver in a dialogue about what is important to him/her
- Assesses extent to which a patient is able to meet/exceed their individual goals and also assess this consistently across a population\(^4\)

Standard Steps for GAS

1. Define and select a patient identified goal
2. Define goal achievement (targeted / expected outcome)
3. Describe the baseline status
4. Define the better and worse outcomes to complete the scale
5. Measure goal attainment at designated follow-up interval(s) using the scale developed

Standard Approach: 5-Point Scale

Baseline -2
Less than expected

Goal

Expected outcome

-1
More than expected

+1
Most favourable

-2
Most unfavourable

A GAS for haemophilia is currently in development

Goal Attainment Scaling for Life – Hemophilia (GOAL-Hem) will be a hemophilia-specific menu of patient-centered goal areas for use in clinical practice and research

Table 1. Goal areas by category

<table>
<thead>
<tr>
<th>Goal Area Category</th>
<th>Goal Areas</th>
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<tbody>
<tr>
<td>Managing hemophilia</td>
<td>Being able to administer factor</td>
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<td></td>
<td>Medication adherence</td>
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<tr>
<td></td>
<td>Procedure planning</td>
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<td></td>
<td>Following treatment plan</td>
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<td></td>
<td>Weight, exercise, nutrition</td>
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<td></td>
<td>Hemophilia care planning</td>
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<tr>
<td>Hemophilia complications</td>
<td>Bleeds</td>
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<td></td>
<td>Muscle bleeds</td>
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<td></td>
<td>Pain</td>
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<td></td>
<td>Joint problems</td>
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<tr>
<td>Impact on activities</td>
<td>Work attendance</td>
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<td>Attending school</td>
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<td>Career planning</td>
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<td>Engaging in sports</td>
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<td>Leisure activities</td>
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<td>General activities</td>
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<td>Access to resources</td>
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<td>Daily personal care</td>
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<td></td>
<td>Use of assistive devices</td>
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<td>Negotiating health insurance coverage</td>
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<tr>
<td>Impact on emotions and relationships</td>
<td>Feelings of anger</td>
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<td></td>
<td>Feelings of sadness</td>
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<td></td>
<td>Narcotic use</td>
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<td>Substance misuse</td>
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<td>Depression</td>
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<td>Relationship with significant other</td>
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<td>Relationships with friends</td>
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<td></td>
<td>Relationships with family</td>
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<td></td>
<td>Self-esteem</td>
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Constructing goals with the GOAL-Hem – Example

Goal: Start exercise program to improve joint health

- (+2) I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 5x week* and able to stand on my feet >30 min without severe pain
- (+1) I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 3x week* and able to stand on my feet >20 min without severe pain
- **GOAL** (0): I have joint problems that bother me *most days*: however, I am *able to manage* physical/emotional toll with *exercise 2x week* and able to stand on my feet >15 min without severe pain
- **BASELINE** (−1): I have joint problems that bother me *constantly*: however, *it is difficult to manage* physical/emotional toll with *physiotherapy and/or pain management*
- (−2) I have joint problems that bother me *constantly*: however, I am *unable to manage* physical/emotional toll with *physiotherapy and/or pain management and have no desire to do so*
GAS scoring standardizes individual goals across different patients

- Scores are effectively the sum of goal attainment $X$ the relative goal weights transformed into a standardized (normally distributed) measure (T-score)$^4$

- Overall scoring returns summary score of 50 when all goals are attained (individual goal attainment = 0)$^7$

$$\text{Overall GAS} = 50 + \frac{10 \sum (W_i X_i)}{\sqrt{(0.7 \sum W_i^2 + 0.3 (\sum W_i^2))}}$$

We need to better utilise existing and new outcome measures to drive improved care

- Health systems should provide the best possible health outcomes while utilising available resources in the most efficient way.
- If the right outcomes are measured and a procurement system is centred around these, improved efficiency (i.e. outcomes for resource used) could be achieved through personalized care.

Example of procurement on adherence and outcomes performance in diabetes

- If outcomes are improved, the manufacturer (Merck) provides a discount to the payor (Cigna) at the end of the year on their oral diabetes drug (Januvia and Janumet).
- Outcomes measured are blood sugar levels (through A1C lab tests) and adherence (through claims data).
  
- Blood sugar levels dropped by 5%
- Adherence increased by 87%

“Cigna stressed that a key factor in this success was the alignment of payer and manufacturer incentives to support better outcomes.” Neumann et al (2011)

Procurement on price and volume does not create right incentives to improve care and value for money

In the current procurement paradigm two levers exist to address efficiency and sustainability:

Current paradigm: Price per IU

- Reduce utilization
  - May only be possible if patients are not optimally treated
  - Risks worsening patient outcomes
- Reduce price
  - One-off mechanism not addressing long term growth
  - Potentially reduces access to innovation for patients

Future efficiency and sustainability requires a new approach to procurement of haemophilia care
Outcomes data collection is critical to measuring and improving system efficiency.

Outcomes based procurement could better align incentives across all stakeholders

<table>
<thead>
<tr>
<th>Patients</th>
<th>Healthcare Professionals</th>
<th>Payer/National Healthcare Systems</th>
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<tbody>
<tr>
<td>Strengthened physician/patient dialog and patient adherence: concordance</td>
<td>Added tracking/monitoring system development and utilization</td>
<td>Explicit, public commitment to improved patient outcomes</td>
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<tr>
<td>Empowerment over disease and its treatment</td>
<td>Improved benchmarking capabilities</td>
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<td>Fewer bleeding events causing long-term chronic joint damage</td>
<td>Increased availability of cumulative real world data</td>
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<td>Improved outcome and QOL</td>
<td>More information available to enable tailored dosing regimens</td>
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<td>Transparency: costs and utilization patterns</td>
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<td>Cost sustainability, predictability, and value for money</td>
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Summary and Conclusions

• There is a need for more individualized outcome measures including patient treatment goals
• These should be complementary to existing clinical and patient reported outcomes, and useful in clinical practice and research
• New and existing outcomes could be used as a basis for procurement to incentivize optimal care in a financially sustainable way